



## Act for ALS EAPs Shared Learnings Workshop Speeding up EAPs from announcement to last patient dosed Key Take-Aways

### Overall take-aways:

- The first ACT for ALS EAPs has provided benefits to some patients and learnings for PIs/sites. Drug sponsors have extracted significant insights into drug efficacy that have not emerged from previous trials, and that might move drug development and approval more quickly.
- Unlike clinical trials, participant recruitment does not slow down the EAP process. Demand exceeds supply. Subcontracting is a barrier that slows the process.
- We need to establish a constant feedback loop to provide lessons learned and models of success to next grantees.
- Work to build a universal model for future EAPS. Any level of standardization will help.

### Top recommendations:

- Create standardized/leverageable site contracts.
- Achieve balance between two virtues of speed and broad access.

### Identifying and activating sites:

- Funding for subawards should flow faster and more efficiently to eliminate both bottlenecks and risk to smaller sponsors.
- Sites experienced in EAPs (for any therapeutic area) will be more efficient than first-timers. Start with sites that either participated in the drug sponsor's phase 1 and/or 2 trials as well as sites that have done existing EAPs. This should provide a running start. Add sites in the next phase to broaden geographic availability.
- Remote sites are needed to provide EAPs to a representative population of people with ALS, however they face challenges that can slow down the process. Providers with experience and infrastructure for remote care, such as Synapticure, should be utilized.

### Enrolling patients:

- Willingness of patients to participate is not an issue, but patients do need help identifying how to access EAPs and which ones are best suited for them. (See Communication)
- Different sites use different methods for selecting participants; however, fairness is a key consideration.



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### Executing EAPs:

- Efficient delivery of EAP treatments across all sites requires good coordination.

### Resources:

- Request NINDs to focus on quicker payment processing to sites once engaged.
- Continue to fund EAPs to supply adequate site staff.
- Designated site staff is necessary for sites with multiple participants.

### Communications:

- Develop a central database and website – administered by an independent group – that could reflect status, sites, information for people with ALS and caregivers in one spot. Communications in the eyes of a person with ALS or caregiver are essential. It's about them.
- The process for applying for an Act for ALS EAP needs to be clear to everyone. If there are waiting lists or other selection criteria because of high demand, the rules need to be clear and fair.

### Research requirements and value:<sup>1</sup>

- Recognize the need for clinical trial quality data for research and encourage FDA to recognize this quality/info in considering accelerated approvals, as they have in oncology?

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<sup>1</sup> Examples of the demonstrated research value from these EAPs was presented at the following event: NEALS Webinar: CNM-Au8® in ALS - NIH Expanded Access Program (EAP) Clinical & Biomarker Update; February 25, 2026



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### APPENDIX – ABBREVIATED CHAT COMMENTARY FROM ZOOM

2026-01-30 15:26:06 From Bob Hebron ALS PS to Everyone:

Would it help with the central group at sites if they were given a time limit to finalize the contract or you will move on to the next site possibility

2026-01-30 15:28:33 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:

Replying to "Would it help with the central group at sites if t...":

Bob, that would probably decrease the number of sites that are interested in offering EAPs. It would be a huge risk to embark on one of these, spend the time (and money) at a site, then miss the deadline and get nothing out of it.

2026-01-30 15:30:13 From Andrea Pauls Backman - ALS Strategy Consulting to Everyone:

Can I very briefly share the results of a survey we ran through NEALS last summer? We gathered data on how clinical sites perceived the delays and successes of EAPs.

2026-01-30 15:39:39 From Kuldip Dave (ALS Association) to Everyone:

I also have a question for the Q&A session later, if each sponsor can talk about ONE, just ONE, outcome measure or biomarker that they measured in the EAP which they were not able to do in the double blind trial (for example, you cannot mention nfl if you also measured nfl in the double blind trial)

2026-01-30 15:43:47 From James Berry to Everyone:

Interesting question, Kuldip. From my perspective, I don't think there are any outcomes for EAPs that can't be measured in an RCT. I don't think that's something the EAPs are trying to achieve. But the population is different. You can't measure NfL in ANYONE with VC <50% in a trial, if the trial excludes people with VC <50%. So, rather than facilitating a different set of endpoints, it allows exploration of similar endpoints in a different, or more generalized, population. In addition, the funding mechanism requires that the data be shared - that is not always the case for RCTs.

2026-01-30 15:47:10 From Kuldip Dave (ALS Association) to Everyone:

Okay, James, so I will pivot and ask, in any of the EAPs funded so far, was there anything learned using the same outcome measure but in a different population which allowed the sponsor to make a decision on designing their next trial better?

2026-01-30 15:47:57 From Troy Fields ALS PS to Everyone:

Replying to "I also have a question for the Q&A session later, ...":



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Perhaps we can add to the question what was learned about patient outcomes and biomarkers by studying patients that don't qualify for trials since that is also new and relevant data.

2026-01-30 15:48:35 From Jess Rabourn to Everyone:

We also conducted a massive Home-Health component in the SEANOBI-ALS EAP

2026-01-30 15:48:43 From Kuldip Dave (ALS Association) to Everyone:

Replying to "I also have a question for the Q&A session later, ...":

Thanks Troy, and whatever was learned helped the sponsors make their next trial better

2026-01-30 15:50:28 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:

Replying to "Okay, James, so I will pivot and ask, in any of th...":

<https://invest.clene.com/news-releases/news-release-details/clene-announces-statistically-significant-als-biomarker-results>

2026-01-30 15:51:30 From Troy Fields ALS PS to Everyone:

Replying to "I also have a question for the Q&A session later, ...":

or add key scientific data to be considered for subsequent FDA approval.

2026-01-30 15:52:48 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:

Replying to "Okay, James, so I will pivot and ask, in any of th...":

The fact that NfL dropped in the Clene EAP is potentially HUGE. I believe this is the first evidence we have that any ALS treatment can work in patients who have disease longer or have less function than those who are traditionally enrolled in trials. If the FDA uses this finding as part of new drug approval, this will change everything. Many more companies will want to offer EAPs. It also gives us data we can use to try and convince payors that it works in patients who look different from those in trials.

2026-01-30 15:53:46 From Kuldip Dave (ALS Association) to Everyone:

Replying to "Okay, James, so I will pivot and ask, in any of th...":

Rick, are you talking about the 10 percent reduction of nfl?

2026-01-30 15:54:01 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:

Replying to "Okay, James, so I will pivot and ask, in any of th...":

Yes.

2026-01-30 15:54:06 From Nader Yaghoubi, PathMaker Neurosystems to Everyone:



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Related to leveling the playing field, what might be done to make the EAP program inclusive of non-pharmacological approaches to ALS, such as what we at PathMaker Neurosystems are developing with multi-site direct current stimulation, a novel form of neuromodulation now in clinical trials for the treatment of ALS? ACT for ALS legislation currently restricts eligibility to drugs and does not provide a level playing field. We have many people with ALS asking about EAPs.

2026-01-30 15:54:11 From Kuldip Dave (ALS Association) to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
okay

2026-01-30 15:54:48 From Lynn Brielmaier ALS PS to Everyone:  
My clinic, Houston Methodist Neurological Institute, started by Dr. Appel in 1981, does not participate in EAPs at all, due to Staff Involvement.  
They do have RCTs.

2026-01-30 15:55:30 From James Berry to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
This is a really important question. For the RAPA program, understanding the immune system changes in people later in the disease and the immune function impacts later in the disease are really impacting the way the clinical development program could be framed.

2026-01-30 15:57:55 From Kuldip Dave (ALS Association) to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
That's great James, that's what sponsors or PI's have to talk up. We were able to collect XXX data that was not available to us in the RCT or we collected XXX data which replicated the RCT data. Otherwise, we are left talking about how many people got access to EAPs and that is not very helpful given we sold EAPs as research to Congress.

2026-01-30 15:59:47 From Nadia Sethi to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
@James Berry does this support opening trials to a broader, more late stage population?

2026-01-30 15:59:56 From Dina Lyon (NIH/NINDS) to Everyone:  
Jinsy makes a great point - we've worked with our colleagues the clinicaltrials.gov to create records especially customized for these NIH-funded EAPs. There are many statutory requirements we have to meet in posting & reporting.



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2026-01-30 16:02:04 From Kuldip Dave (ALS Association) to Everyone:  
so can that communications happen from a site built by NIH ? NEALS?

2026-01-30 16:02:08 From Andrea Pauls Backman - ALS Strategy Consulting to Everyone:  
The idea of an participant-facing EAP communications portal would be very helpful to the ALS community. Is this something that could be added to the AMP-ALS portal?

2026-01-30 16:02:16 From James Berry to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
Perhaps another bent on your original question, Kuldip, is not so much, "What can be done in an EAP that can't be done in a trial?" But rather, "What can be done in an EAP that can't be done in an observational study?" That's more nuanced. While the same outcomes can be tested, dropout is a bigger problem in observational studies. And selection bias is different in observational studies. Progression rates in EAPs are more representative than observational studies (tend to be slower). So, that's an opportunity to learn about the disease, too.

2026-01-30 16:04:43 From James Berry to Everyone:  
Replying to "Okay, James, so I will pivot and ask, in any of th...":  
Another great question, Nadia. Maybe - it depends on the structure and purpose of the trial and trial intervention. Yet we want to keep in mind that the main goal of trials is determine efficacy as quickly as possible. So, enrolling a homogeneous population can be a really important tool for trials. (This is, perhaps, why EAPs have a role.)

2026-01-30 16:05:12 From Kuldip Dave (ALS Association) to Everyone:  
I thought the IAMALS signal site had list of EAP's, no?  
or the ALS TDI trial list site?

2026-01-30 16:06:18 From Nadia Sethi to Everyone:  
Replying to "or the ALS TDI trial list site?":  
This one does include EAPs listed on ct.gov.

2026-01-30 16:07:49 From Jinsy Andrews, MD, MSc to Everyone:  
Actually My Tomorrows has a clinical trial concierge service that could potentially be the concierge for this type of information

2026-01-30 16:08:00 From James Berry to Everyone:



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Apologies - I have to step away. Really important and good conversation. Thank you for organizing this, Problem Solvers!

2026-01-30 16:08:49 From Kuldip Dave (ALS Association) to Everyone:  
Replying to "Actually My Tomorrows has a clinical trial concier...":  
Yes, and they include EAPs in their concierge service, i think  
<https://mytomorrows.com/contact-us-als-patients/>

2026-01-30 16:09:10 From Jinsy Andrews, MD, MSc to Everyone:  
I've seen other disease advocacy nonprofit organizations help communicate pipelines and research to community really well like Cure SMA .

2026-01-30 16:09:13 From Melanie Lendnal to Everyone:  
@Jinsy Andrews, MD, MSc Stephanie from myTomorrows was at the recent CUPA event in Boston and we spoke about this. She is interested in exploring this as a possibility.  
<https://www.curesma.org/sma-drug-pipeline/>

2026-01-30 16:09:36 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:  
Replying to "Actually My Tomorrows has a clinical trial concier...":  
Great! We should look into this. I've never heard of anyone using it. Would love to get feedback from those who used it.

2026-01-30 16:10:33 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:  
Replying to "Actually My Tomorrows has a clinical trial concier...":  
If it really is helpful, I would share it every day with all the folks who email me looking for guidance

2026-01-30 16:11:30 From Jess Rabourn to Everyone:  
It might be worth ensuring that invited members of the proposal review groups be better informed about the difference between EAPs and clinical research trials. One of the comments we received was that our targeted enrollment was "greater than necessary" to meet the requirement of statistical significance, which is inconsistent with the spirit and regulation of Expanded Access.

2026-01-30 16:12:05 From Jinsy Andrews, MD, MSc to Everyone:  
PPMD is another non profit for duchenne muscular dystrophy that helps to communicate about research progress, trials and newly approved therapies:



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<https://www.parentprojectmd.org/research/>

2026-01-30 16:17:27 From Jinsy Andrews, MD, MSc to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

IAMALs does have an EAP listing but it is dependent on what is available on clinical trials.gov

2026-01-30 16:18:13 From Kuldip Dave (ALS Association) to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

so should NIH require as part of their granting that sponsor create a listing on ct.gov?

2026-01-30 16:18:57 From Abigail O'Connell (Synapticure) to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

It is required that each study is listed on clinical trials.gov

2026-01-30 16:19:17 From Jinsy Andrews, MD, MSc to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

It is required to have a listing but the internal infrastructure is different than a clinical trial listing

2026-01-30 16:19:22 From Nadia Sethi to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

All the trial finders only show info on clintrials.gov. And a lot of times that info is not correct.

2026-01-30 16:19:43 From Nadia Sethi to Everyone:

Replying to "I thought the IAMALS signal site had list of EAP's...":

And that goes for MyTomorrows as well, having talked with them.

2026-01-30 16:19:52 From Kuldip Dave (ALS Association) to Everyone:

going back to Rich Bedlack's point on more demand than spots for EAP, is there a way to set up a lottery system to make it more fair?

2026-01-30 16:20:15 From Jess Rabourn to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":

Please don't do that.

2026-01-30 16:20:30 From Kuldip Dave (ALS Association) to Everyone:



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Replying to "going back to Rich Bedlack's point on more demand ...":  
why not?

2026-01-30 16:20:54 From Jess Rabourn to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
Lotteries restrict access. Takes focus away from scale.

2026-01-30 16:21:10 From Andrea Pauls Backman - ALS Strategy Consulting to Everyone:

I'd still like to quickly share some findings from our NEALS survey on this topic.

2026-01-30 16:21:22 From Rick Bedlack ALS Doc, Researcher, Advocate to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
Agree with Jess. This would markedly decrease our efficiency as far as contacting and enrolling patients

2026-01-30 16:21:30 From Kuldip Dave (ALS Association) to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
I don't understand how that happens

2026-01-30 16:22:04 From Jess Rabourn to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
Call me

2026-01-30 16:22:07 From Abigail O'Connell (Synapticure) to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
At Synapticure we did use a lottery system after extensive discussion with Clene, Dr. Andrews, and NINDS

2026-01-30 16:22:29 From Kuldip Dave (ALS Association) to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
Abigail, did it reduce your efficiency?

2026-01-30 16:24:13 From Lynn Brielmaier ALS PS to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":  
most PLWALS already feel like losers  
in the health lottery  
Don't need it amplified yet again



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2026-01-30 16:25:32 From Abigail O'Connell (Synapticure) to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":

No, we felt that while there's no perfect way to approach enrollment when demand outstrips the available spots, it was the most equitable way to approach enrollment. Our sense is that employing a first-come, first-served approach inherently disadvantages participants who aren't already "in the loop" and connected/aware of research options, and the reason Synapticure was included in the Clene EAP was to expand access to a broader patient population

2026-01-30 16:29:34 From Nadia Sethi to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":

From having engaged with thousands of people over the last 7 years... there is so much inequity in all research and access. Lottery sounds better than giving access first and foremost to those have means to get to sites first, or travel, or so on.

2026-01-30 16:30:40 From Kuldip Dave (ALS Association) to Everyone:

Replying to "going back to Rich Bedlack's point on more demand ...":

Exactly my point Nadia. Otherwise, if you know Merit or Jinsy (and I love them both) are you better able to get into an EAP versus someone not named Merit or Jinsy

2026-01-30 16:39:31 From Jess Rabourn to Everyone:

We gave time limits to prospective participating sites

2026-01-30 16:40:47 From Jess Rabourn to Everyone:

They were internally incentivized to achieve these timelines and have communicated to us that this exercise has made them much faster for next time

2026-01-30 16:43:52 From Jess Rabourn to Everyone:

We had multiple back-up sites to bring in if any of the early round invitees bogged down. Not every institution is prepared to move an EAP through its internal approval process.

2026-01-30 16:44:17 From Kuldip Dave (ALS Association) to Everyone:

all of the nonprofits on this call who give out grants can give 5 day lectures on how long contracting and legal back and forth takes and how its death by 1000 needles

2026-01-30 16:51:41 From Melanie Lendnal to Everyone:



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what Kuldip is saying is critically important as we work with MOCs on the Act for ALS reauthorization.

2026-01-30 16:51:48 From Troy Fields ALS PS to Everyone:  
excellent request Kuldip

2026-01-30 16:55:40 From Marjan Sepassi (Clene Nanomedicine) to Everyone:  
Replying to "excellent request Kuldip":  
and hopefully new abstracts will be accepted very soon

2026-01-30 16:55:46 From Kuldip Dave (ALS Association) to Everyone:  
Replying to "excellent request Kuldip":  
We need a bullet/slide from each of the EAP programs (not just Clene)

2026-01-30 16:56:53 From Lynn Brielmaier ALS PS to Everyone:  
Replying to "going back to Rich Bedlack's point on more demand ...":  
I have been personally denied an EAP.  
I know what it's like to be on the outside looking in.